ERANET Neuron

How can the lay organizations in the field of neurology work with the basic scientists and the clinicians to fight neurological disorders?

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EFNA
European Federation of Neurological Associations
Empowering Patient Neurology Groups

RESEARCH

BEFORE

- Assessing Unmet Needs
- Setting Patient Relevant Endpoints/Outcomes
- Devising Patient Friendly Methodologies
- Aiding Recruitment

CLINICAL TRIALS

AFTER

- Disseminating Results
- Advocating for Access/Reimbursement
- Shaping/Reshaping policy and health care delivery

TOGETHER UNDER THE UMBRELLA

an initiative of

EFNA

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Patient-Public Involvement: Our Role

The ‘Healthy Brain Healthy Europe’ conference led by the Irish Presidency in 2013 recommended the promotion of:

“The role of patients in all stages of research and evidence-based healthcare… Patients need to be actively involved in the planning of research approaches, the execution of services and the maintenance of standards of healthcare.”

Patient-Public Involvement: How?

In the UK, a requirement for PPI is universal for funding applications in translational research

BUT

There is variability across the EU on whether PPI is required to secure national government/charitable funding.

Whether it is mandatory or not – there is widespread agreement there are challenges but that it is, ultimately, beneficial!

Patient-Public Involvement: The Benefits

- Helping identify and prioritize the most patient-relevant topics
- Identifying cultural issues that should be taken into account
- Suggesting the best way to get informed consent from patients and/or carers
- Ensuring that information sheets, questionnaires and interview schedules are patient-friendly
- Speeding recruitment by providing researchers with better access to the patient community
- Helping to disseminate results through patient-led advocacy, relating research findings to patients' own experience and presenting them in a more user-friendly manner

Remember: Research protocols (including methodologies and eventual outcomes/endpoints) which are planned with inputs from patients are more likely to work and succeed in the 'real world'.

Patient-Public Involvement: The Challenges

- Deciding the most appropriate type of PPI: e.g. young people with MS vs. elderly people with dementia

- Ensuring diversity of representation

- Appropriately compensating patients without affecting their welfare/disability payments, etc.

- Speaking the same language

- Potential conflicts between what patients feel are in their best interest and the scientific process – for example, including a placebo arm in a trial

- Lack of experience, education and awareness – *on both sides!*

Patient-Public Involvement: How?

- Need to educate patients and scientists on why PPI in research is necessary and how it can be done

    - Empower patients/society to understand and participate in the scientific process
    - Highlight patient needs and concerns to scientists

- PATIENT ORGANISATIONS CAN BE KEY PARTNERS
    - Obvious first point of contact
    - Conduit between patients and researchers
    - Provide capacity-building courses to patient advocates (e.g. EUPATI, EURORDIS Summer School)
    - **EFNA currently developing Training Initiative for Neurology Advocates [TINA] Expressions of Interest Welcome!**

Patient-Public Involvement:

Why most important in neurology?

- Most impactful symptoms and disease progress often cannot be directly measured: must be assessed using questionnaires or interviews with patients
- For many disorders, regulators now require evidence that a drug changes the patient’s real-world functioning as well as improving symptoms.
- Secondary symptoms often more burdensome than primary/Multi-Morbidity
- Clinical scales against which outcomes can be measured are, therefore, limited.

SO COULD WE PARTNER TO FIGHT NEUROLOGICAL DISORDERS BY:

Encouraging/facilitating patient participation in clinical trials (not just as participants), and ensure patients can access high quality information before and after

Create and evaluate new clinical scales which take a more holistic approach and measure patient relevant outcomes?

Establish and manage pan-European data registries to create real world data?

Working Together to reach Decision/Policy-Makers

BRAIN
MIND
PAIN

Member of the European Parliament Interest Group

HELP US TO SET THE AGENDA

MEP Interest Group on Brain, Mind and Pain

Aims to: Encourage research into and access to innovative treatments, promote prevention and self-management approaches, decrease stigma and work together to improve quality of life for people living with these disabling conditions.

- Support patient-led campaigns to educate, eradicate stigma and raise awareness of neurological and chronic pain disorders
- **Support research into the development of innovative prevention and treatment options within a regulatory framework which facilitates equitable access to affordable therapies**
- **Strengthen patient involvement in this research, and in policy setting and decision-making**
- Implement relevant European social legislation to ensure appropriate support for people living with neurological and chronic pain disorders

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Working Together: What next?

We need to work together to convince policy-makers of the importance of funding research into neurological disorders – as a whole – starting with mutually agreed areas of greatest unmet need.

National/European funders of research should provide guidance on the appropriate engagement of patients in different types of research, and support the development of national/European support services to assist researchers in this engagement.

All players need to be involved in setting and agreeing ‘the rules of the game’ via multi-stakeholder roundtables.

We need to partner to create tools to generate the most patient relevant outcomes e.g. data registries, clinical scales, etc.

Templates exist but we need a specific, tailored framework for the field of neurological disorders.

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The Book of Evidence can be accessed at: www.brainmindpain.eu